

PUBLIC WITNESS TESTIMONY
HOUSE APPROPRIATIONS SUBCOMMITTEE ON
LABOR, HEALTH AND HUMAN SERVICES AND EDUCATION

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NEW YORK, NEW YORK

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2:00 PM – 4:30 PM

PRIMARY IMMUNODEFICIENCY

Mrs. Modell will be discussing both the education and awareness program and the newborn screening activities related to Primary Immunodeficiencies in the Centers for Disease Control and Prevention (CDC) in the Department of Health and Human Services.



Jeffrey Modell
Foundation

Curing PI. Worldwide.

Testimony of Vicki Modell for the Jeffrey Modell Foundation
House Appropriations Subcommittee on
Labor, Health and Human Services and Education
May 12, 2010

Mr. Chairman and Members of the Subcommittee:

Thank you for the opportunity to present this testimony to the Subcommittee. My husband Fred and I created the Jeffrey Modell Foundation in 1987 in memory of our son, Jeffrey, who died at the age of 15 as a result of a life long battle against one of the estimated 160 primary immunodeficiency (PI) diseases.

The Jeffrey Modell Foundation is an international organization with its headquarters in New York City. In the 24 years since we established it, the Foundation has grown into the premier advocacy and service organization on behalf of people afflicted with primary immunodeficiency diseases. As a demonstration of the extent to which the JMF leads in the field, please consider the following:

- The Foundation has created Jeffrey Modell Research and Diagnostic Centers at 72 academic and teaching hospitals from coast to coast in the United States and throughout the world. They are located on every continent. In addition, we are affiliated with more than 415 referring physicians at 171 academic medical centers in 59 countries and 169 cities, again located on every continent throughout the world.
- The Foundation conducts a National Physician Education and Public Awareness Campaign, currently funded with approximately \$3.1 million appropriated by this subcommittee to the Centers for Disease Control and Prevention (CDC) and awarded by competitive contract to the Foundation. **To date, the Foundation has leveraged the federal money to generate in excess of \$125 million** in donated media with hundreds of thousands of placements on television, radio, print, and other public media, as well as a 30-minute program produced for PBS. The Campaign has also included physician symposia, conducted for CME credits in locations throughout the country. It has included mailings to physicians in a variety of specialist and primary care fields, including pediatrics and several pediatric specialties, family practice, and internal medicine, as well as school nurses, clinical and registered nurses and daycare centers.
- In addition, the Jeffrey Modell Foundation has been the leader in advancing newborn screening for some of the most severe forms of PI. Working with CDC, NIH, UCSF and private industry, we helped fund the development of a newborn screening test that was pilot tested in Wisconsin. The results were so successful that Wisconsin and

Massachusetts have now implemented population-based screening of every baby born in their states. Then, in January of this year, we were successful in having the Secretary's Advisory Committee for Children with Heritable Disorders add this test to the core panel of 29 newborn screening tests recommended for the states to utilize. It is the first test to be added since the core panel was created in 2005. The test is already saving lives and we know that as more states adopt it, many more will be saved.

First and foremost, Mr. Chairman, we want to thank you and all the members of this subcommittee on both a personal and a professional level. Personal because whenever we come to Washington, whether it is to testify here before the committee or to meet with the members of the subcommittee individually in their offices, every Member of Congress and every member of your staffs are unfailingly polite, courteous, interested and caring. The warm and understanding response that we receive makes this a labor of love for us.

And, professional because over the twelve years that we have been coming to Washington, we have been given the opportunity to build a partnership with the Congress, the Centers for Disease Control and Prevention, the National Institutes of Health, as well as with our own supporters in the private sector, including industry and other concerned donors. We believe that we have maximized the benefits for patients from the support that this subcommittee has afforded us. I would like to take a few minutes to discuss where we are, where we are going with your continued support, and some changes that are needed in the President's budget request to help us help patients.

PI Education and Awareness Program

This subcommittee is currently providing CDC with **\$3.1 million** for physician education and public awareness of immunodeficiencies for FY10. This is part of an overall budget of **\$12.3 million** for the Office of Public Health Genomics, which uses the remaining \$9.2 million for its operations.

Since the Campaign's inception, it has generated more than \$125 million in donated media, including television and radio spots, magazine ads, billboards, airport signs and other print media. It has also enabled us to generate additional funding from the private sector – both individuals and the pharmaceutical industry. To this point, **every \$1 of federal funds provided by the subcommittee to this program has been leveraged into more than \$10 for this education and awareness program.**

Most importantly, Mr. Chairman, I am delighted to report to you that the program that this subcommittee has funded is having exactly the impact that all of us hoped it would when it was created. Allow me to give you some specifics.

Surveying the physicians at the Jeffrey Modell Centers Referral Network we have learned that **the number of patients referred, diagnosed and treated has doubled every year** since the program's inception. The negative health outcomes of undiagnosed cases – **infections, hospital and physician visits, and similar costs – decreased an average of 70 percent** for diagnosed patients.

But, it is fair of this subcommittee to ask “so what?” What difference does it make to the health of these patients if they are now in treatment? What is the real impact in a real world sense on the patients that are found?

The economic impact of PI diagnosis has been carefully assessed comparing the costs of treatment before diagnosis and after. In round numbers what we learned was that the average annual cost of health care for an undiagnosed patient is \$103,000 per year. The same costs for the same patients in the year after diagnosis are \$23,000. **The gross annual savings to the health care system is \$80,000 per patient.**

Mr. Chairman, this program is working and we are delighted. But this is where the problem comes along. The President’s budget for FY11 reduces funding for the Office of Public Health Genomics from \$12.3 million to \$11.7 million. Further it eliminates the line item created by this subcommittee to fund the education and awareness program. While CDC has indicated its support for continuing the program, the only guarantee that will happen is if you act.

For this reason, we are asking that you take three modest steps as you are assembling the Chairman’s Mark for the bill:

- **First, restore the total line item for the Office of Public Health Genomics to its FY10 level of \$12,308,000.**
- **Second, break that money out into two separate lines, as its now – \$9,201,000 for the Office and \$3,107,000 for PI Education and Awareness.**
- **Third, so that there is no misunderstanding, include a paragraph of Committee Report language that says:**

The Committee believes that the education and awareness program for primary immunodeficiencies has been a model of public-private cooperation and therefore has restored the current structure for the Office of Public Health Genomics budget. The program’s success in leveraging public money for private investment has resulted in a huge return on the federal dollar, led to reduced health disparities, and will save lives as the program directs greater attention to newborn screening.

Newborn Screening Program

As described above, early diagnosis is critical to the health of patients and to saving the health care system money. And, there are few better examples of early diagnosis than newborn screening. The JMF has worked long and hard to support the development of a newborn screening program for some of the most severe and deadly forms of PI.

Early detection of these diseases through newborn screening is critical because bone marrow transplants cure over 98 percent of infants who have the procedure before developing any serious

infections. The treatment costs less than \$10,000. However, if an infant receives a transplant after developing severe infections, the success rate is only between 60 and 70 percent; the costs associated with the treatment of these infants can be as high as \$1 million over their lifetime.

As described above, the Secretary's Advisory Committee on Children with Heritable Disorders has recommended to the Secretary that this test be added to the core panel that forms the basis of newborn screening in states throughout the nation. It is the first time the list has ever been amended since it was created five years ago. The Jeffrey Modell Foundation is proud to have played a role in this advancement for babies and we are urging the Secretary to accept the recommendation promptly.

Once she has done so, newborn screening officials in numerous states have advised us that they will move forward with including this test in their states. At that time, the Foundation is committed to moving forward with the production of educational materials for state labs and families that will provide the information they need to consider the results of the test their baby is having. **The funds for the education and awareness program are critical for making the most of this important improvement in public health.**

Conclusion

With the support the Jeffrey Modell Foundation has received from this subcommittee over the years, we have been able to increase the public's awareness of PI and most importantly improve and save lives. We are grateful for your past and continued support. While we understand that the subcommittee must make difficult decisions in this fiscal environment, please remember that the Foundation has successfully leveraged federal dollars to expand the reach of all of our activities. Frankly, the collaboration between the federal government and the Jeffrey Modell Foundation has been a model for successful public-private collaborations. The impact of every federal dollar spent on the education and awareness campaign and on newborn screening has been exponentially increased by our commitment to bring the Foundation's resources to bear.

We ask again that you restore the funding to FY10 levels; break out PI Education and Awareness into a separate line item; and include the report language provided to assure that this program maximizes its impact.

Mr. Chairman, again, we are delighted to have the opportunity to present to the subcommittee and stand ready to work with you.